

Wednesday 30 May 2018



# GUARD-Heart

Gateway to Uncommon And Rare Diseases of the Heart



ERN GUARD-HEART BIMONTHLY NEWSLETTER

YEAR 2018 NUMBER 3

## 4<sup>th</sup> EU Conference on the ERNs “ERNs in Action”

European Commission, Brussels, 25 May 2018

The 4<sup>th</sup> Conference on the European Reference Networks “ERNs in action”, organized by the European Commission DG SANTE, will take place on 21&22 November 2018 in Brussels. The Conference organisation is ongoing. In the upcoming weeks the EU will send a formal invitation and the agenda.

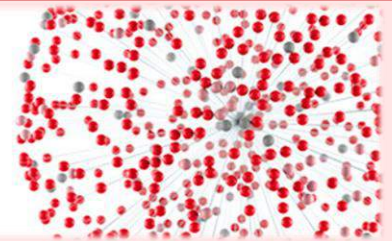
EU will invite and cover travel and accommodation expenses for 10 participants per ERN. At this stage, the EU asks each ERN to appoint 10 representatives from the Network. It is important that ERN GUARD-Heart timely provides the names and contact details of the 10 persons who will represent the ERN at this Conference. This information is important as the EU needs to book travel and accommodation a.s.a.p in order to secure the hotel rooms and direct flights.

**The deadline for submission of the lists of participants is 8 June 2018.** So members of the 24 HCPs of ERN GUARD-Heart are requested to inform the project management office a.s.a.p. whether they wish to participate ([n.hofman@amc.nl](mailto:n.hofman@amc.nl)). Since the number of invitees will be limited, one member per HCP will be accepted (with a preference for the HCP representative member) and the registration will be confirmed on a first-come-first-serve basis. ♥

### ESC Congress Munich 2018

25-29 August

Where the world of  
cardiology comes together



The 4<sup>th</sup> meeting of the ERN GUARD-Heart Board will be held on 28 August 2018, 9:00-12:30 (followed by lunch), in Munich, Germany, during the annual European Society of Cardiology Congress. Further details on the location of the meeting will be announced soon.



ERN members who wish to discuss a topic or provide a presentation can contact the project management office. The agenda of the meeting will be disseminated among the members around mid August 2018.



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## ERN-related conferences and meetings

### Cardiac Hereditary Diseases Emergency Institute for Cardiovascular Diseases "Prof. Dr. C.C. Iliescu"

**Bucharest, 3 April 2018**

On 3 April 2018, an educational course on genetics and cardiac hereditary diseases (including electrical diseases, cardiomyopathies, & paediatric conditions) was organized at the Emergency Institute for Cardiovascular Diseases "Prof. Dr. C.C. Iliescu" (Bucharest, Romania). The course directors were Dr. Ruxandra Jurcut & Prof. Carmen Ginghină. Ruxandra Jurcut is a representative member of ERN GUARD-Heart and the course was endorsed by the Network. In addition, at the meeting, the release of a study book on genetic cardiomyopathies, by the course directors, was announced. ♥



Ruxandra Jurcut (left) and Carmen Ginghină (right) at the Cardiac Hereditary Disease Course in Bucharest.

## SAVE THE DATE

**Educational Course on Sudden Cardiac  
Death & Familial Electrical Diseases**

**6 December 2018 – London, UK**



Co-funded by  
the European Union



### ERNs and Clinical Research Workshop by RD-ACTION, DG SANTE & European Medical Agency

**London, 29 May 2018**

On 29&30 May, RD-ACTION, in collaboration with the DG SANTE and the European Medical Agency (EMA) organizes a workshop on "How can ERNs add value to clinical research in rare diseases and highly specialised domains?" at the EMA in London, UK. The principal aims of this workshop are to share the state of the art of tools and resources to optimise clinical research, to better understand the priorities & needs of the ERN community specific to clinical research, and to elucidate the services & opportunities offered by the EMA. ERN GUARD-Heart will be represented by Andrea Mazzanti, cardiologist from the Clinical Scientific Institutes Maugeri IRCCS (Pavia, Italy). ♥

RESEARCH DOMAINS  
PERSONALISED MEDICINE



### Therapeutic Innovation in Inherited Cardiovascular Conditions Symposium

**Friday 8 June 2018**

**UCL Great Ormond Street Institute of Child  
Health, London, UK**

for more information visit [website](#)





## ERN-related conferences and meetings

### European Conference on Rare Diseases & Orphan Products

Vienna, 12 May 2018

The European Conference on Rare Diseases & Orphan Products (ECRD), organised by EURORDIS, took place on 10-12 May 2018 in Vienna. ERN GUARD-Heart was represented by members of two patient organisations involved in the ERN: Patricia Vlasman (H4O, Netherlands) and Ester Costafreda (SAMS, Spain).

EURORDIS and the rare disease patient community have worked together for over a decade to develop the concept of European Reference Networks (ERNs) as patient-centred networks. EURORDIS, in collaboration with the European rare disease community, has now established 24 European Patient Advocacy Groups (ePAGs) as forums to optimise the involvement of patient representatives of the rare disease community in the 24 ERNs. At this moment there are >300 patient representatives active within the ERNs. Now that the concept of the ERNs is established, it is time to see what the current healthcare situation in Europe is and what challenges the ERNs are up to. At this moment the healthcare in every single European country is facing a revolutionary change.

The patient of today is:

- overloaded with health information,
- lacking support to understand their choices,
- challenging healthcare professional with new questions and information,
- unaware of how new technologies will impact his/her health, and,
- not involved in the development of the healthcare's future.

The goal of the ERNs would be to establish 'the patient of tomorrow' who is:

- connected (self-monitoring),
- better informed (social media + online diagnosis),
- more engaged in his/her healthcare decisions,
- understands the value of evidence-based medicine
- owns their medical information, and,
- helping to inform a healthcare environment that is ethically and socially responsible.

Importantly, these changes require (new) capacities from healthcare professionals. The healthcare professionals of today are:

- struggling to keep up with evidence-based information,
- lacking support to be better partners with their patients,
- challenged with new information/questions from their patients,
- being faced with new diagnostic tools, and,
- not fully aware of the ethical and social challenges that new technologies may bring.

Taking these points into consideration the ERNs have to attempt to stimulate the so-called 'professional of tomorrow' who are:

- enabled by technology to 'humanize' the way they provide care,
- working in partnership with their patients,
- returning to the 'art' of medicine (not only the technology),
- working in 'communities' of health, and,
- helping to inform a healthcare environment that is ethically and socially responsible.

In order to make the transition to this new concept of patient and healthcare provider, the key word is "Patient Partnership". Therefore, the overall slogan to summarize the ECRD 2018 will be: *"Together, I am better"*. ♥

**Text by Patricia Vlasman**



Ester Costafreda (left) and Patricia Vlasman (right) at the ECRD 2018 in Vienna.



## ERN-related activities

### New app for cardiology trials, guidelines and registries

Amsterdam, 28 May 2018



“Hart4Onderzoek”, a patient advocacy group that is participating in the Patient Advocacy Board of ERN GUARD-Heart, is currently developing a Mobile app for healthcare providers to facilitate the inclusion of patients in clinical trials and patient registries. The main goal of the app is to build a bridge between patients and science & to promote the participation of more empowered patients in scientific research. The app will provide healthcare professionals across Europe an up-to-date list of clinical trials and patient registries in the field of cardiology, and will focus on trials and registries on patients with rare cardiac diseases. Healthcare providers can use the app to review available clinical trials & patient registries in Europe & gain detailed information about each specific trial/registry, the inclusion criteria and how to contact the trial/registry coordinators to include patients. Moreover, the app will provide access to clinical guidelines and consensus recommendations which have been identified as best practice tools by ERN GUARD-Heart. More details about the app and its expected date of release will be published in future editions of this Newsletter. ♥

### Periodic technical report of ERN GUARD-Heart submitted

Amsterdam, 30 April 2018

On 30 April 2018, the project management office of the ERN GUARD-Heart submitted a summary report of the activities of the Network throughout the first year (March 2017 - February 2018) to the European Commission. In this document, the activities carried out by the Network and the results obtained by the Network, in relation to its goals & specific objectives, were summarized. In addition, a list of the achieved deliverables and milestones were included. This periodic technical report is available for all members of the ERN GUARD-Heart on the [IT-platform](#). ♥

### Clinical Patient Management System (CPMS)

Brussels, 16 May 2018

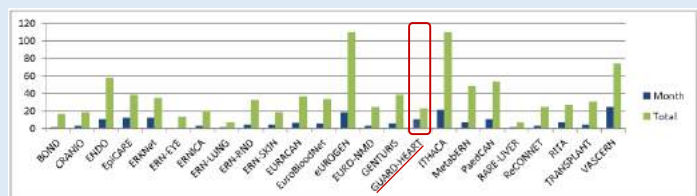
**IMPORTANT NOTICE**

CPMS is the secure online consultation system for second opinion service within the ERNs. CPMS is part of clinical care and approval of a medical ethical committee for research is not required. **However healthcare providers (HCPs) should obtain approval for the use of CPMS for care by their local ethical committees.**

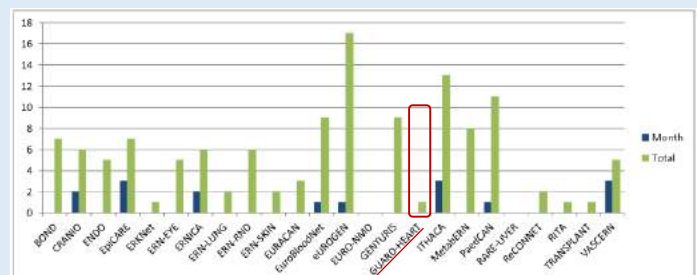
**Each HCP within the ERN GUARD-Heart will be assessed by the European Commission with regard to its activities within the CPMS.**

The first patient for online consultation within ERN GUARD-Heart was discussed in March 2018. The second patient is currently discussed. The ERN has recently been awarded with a grant from the EU to establish a helpdesk to assist all ERN members in using the CPMS. **Please contact the project management office for any assistance to discuss your patient in the CPMS with other experts within ERN GUARD-Heart.**

Figures below show the status report for April 2018, published by the European Commission, with regard to the use of CPMS by each ERN. ♥



Active CPMS users per ERN.



Open patient panels per ERN.



## Highlight



**European  
Reference  
Network**

for rare or low prevalence  
complex diseases

**Network**

Heart Diseases  
(ERN GUARD-HEART)

**Member**

Pediatric hospital  
Bambino Gesù, Rome —  
Italy

# Active participation of the Bambino Gesù Children Hospital in several ERN registries

Rome , 18 April 2018

Upon the publication of a full list of the ERN GUARD-Heart patients registries in the previous edition of this [Newsletter](#), Drs Fabrizio Drago & Anwar Baban have shown great enthusiasm and dedication to include their patients in 5 of these registries, i.e., the ARVC registry, CPVT registry, KCNE1 registry, LQTS registry, and the PRKAG2 registry. Therefore, in this section of the Network Newsletter, we acknowledge Fabrizio Drago and Anwar Baban & encourage other ERN centres to also include their patients in current ERN registries.

Dr Fabrizio Drago is the Head of the Paediatric Cardiology and Arrhythmias/Syncope complex Units at the Department of paediatric cardiology & cardiac surgery of the Bambino Gesù Children Hospital and Research Institute in Rome (Italy). The hospital is a tertiary care centre with over 23 specialties and subspecialties. It serves not only the pool of the Lazio region but most of the central and southern regions of Italy and several children who are referred from overseas with humanitarian missions. This department was established from early 80's with specific mission to manage children with heart diseases including congenital heart defects (>500 open heart surgeries/year), VAD implant (including infants weighing <10Kg), heart transplant (active since 1986), and a dedicated location for CMP and electrophysiology team (>650 ablations/year).

Fabrizio Drago is a well-known paediatric electrophysiologist and an expert in both congenital heart defects and cardiomyopathies. In addition to his qualities as a Head for Cardiology he was a professional Rugby player who did 220 matches in Italian first division.

The Cardiology and Arrhythmology unit works in harmony with other specializations. For instance a dedicated Cardiogenetic clinic is established and run by a cardiogeneticist (Dr Anwar Baban) who is trained both in medical genetics (4 years) and in-ward cardiology medical management (6 years). Anwar Baban is in charge of this clinic since 2015.

The Cardiogenetic clinic includes both inpatient and outpatient service. It manages the following categories: cardiomyopathies, genetically determined arrhythmias, sudden cardiac death, congenital heart defects (isolated and syndromic), and pulmonary hypertension. This clinic is backed up by in house cytogenetic and molecular genetic lab who runs specific custom gene panels dedicated for each above mentioned category. The main aim of this clinic is to offer not only a narrow vision of genetic background but to address patients to personalized multidisciplinary management. After all these full activities, Anwar Baban did not cultivate time for many hobbies except for two passions: learning foreign languages and dedicate time for middle-eastern cuisine. ♥



*Anwar Baban and Fabrizio Drago.*





# ERN GUARD-Heart Publications on PubMed

Amsterdam, 29 May 2018

Official membership of ERN GUARD-Heart can be stated in scientific publications as a separate affiliation using the following text: *Member of the European Reference Network for rare, low prevalence and complex diseases of the heart - ERN GUARD-Heart*. In addition, according to EU regulations, a scientific publication is considered as an ERN article if members from  $\geq 2$  ERN centres are co-authoring on the paper.

Based on these regulations, the next PubMed items are considered as ERN GUARD-Heart publications:

Charron P, Elliott PM, Gimeno JR, Caforio ALP, Kaski JP, Tavazzi L, Tendera M, Maupain C, Laroche C, Rubis P, Jurcut R, Calò L, Heliö TM, Sinagra G, Zdravkovic M, Kavoliuniene A, Felix SB, Grzybowski J, Losi MA, Asselbergs FW, García-Pinilla JM, Salazar-Mendiguchia J, Mizia-Stec K, Maggioni AP; EORP Cardiomyopathy Registry Investigators. The Cardiomyopathy Registry of the EURObservational Research Programme of the European Society of Cardiology: baseline data and contemporary management of adult patients with cardiomyopathies. *Eur Heart J*. 2018;39:1784-1793.

Empana JP, Blom MT, Böttiger BW, Dagues N, Dekker JM, Gislason G, Jouven X, Meitinger T, Ristagno G, Schwartz PJ, Jonsson M, Tfelt-Hansen J, Truhlar A, Tan HL; ESCAPE-NET Investigators. Determinants of occurrence and survival after sudden cardiac arrest - A European perspective: The ESCPAE-NET project. *Resuscitation*. 2017;124:7-13.

Michowitz Y, Milman A, Sarquella-Brugada G, Andorin A, Champagne J, Postema PG, Casado-Arroyo R, Leshem E, Juang JJM, Giustetto C, Tfelt-Hansen J, Wijeyeratne YD, Veltmann C, Corrado D, Kim SH, Delise P, Maeda S, Gourraud JB, Sacher F, Mabo P, Takahashi Y, Kamakura T, Aiba T, Conte G, Hochstadt A, Mizusawa Y, Rahkovich M, Arbelo E, Huang Z, Denjoy I, Napolitano C, Brugada R, Calo L, Priori SG, Takagi M, Behr ER, Gaita F, Yan GX, Brugada J, Leenhardt A, Wilde AAM, Brugada P, Kusano KF, Hirao K, Nam GB, Probst V, Belhassen B. Fever-related arrhythmic events in the multicenter Survey on arrhythmic events in Brugada syndrome. *Heart Rhythm*. 2018. pii: S1547-5271(18)30346-1.

O'Mahony C, Jichi F, Ommen SR, Christiaans I, Arbustini E, Garcia-Pavia P, Cecchi F, Olivotto I, Kitaoka H, Gotsman I, Carr-White G, Mogensen J, Antoniadis L, Mohiddin S, Maurer MS, Tang HC, Geske JB, Siontis KC, Mahmoud K, Vermeer A, Wilde A, Favalli V, Guttman O, Gallego-Delgado M, Dominguez F, Tanini I, Kubo T, Keren A, Bueser T, Waters S, Issa IF, Malcolmson J, Burns T, Sekhri N, Hoeger CW, Omar RZ, Elliott PM. An international external validation study of the 2014 European Society of Cardiology Guideline on Sudden Cardiac Death Prevention in Hypertrophic Cardiomyopathy (Evidence from HCM). *Circulation*. 2018;137:1015-1023.

Schwartz PJ, Ackerman MJ, Wilde AAM. Channelopathies as causes of sudden cardiac death. *Card Electrophysiol Clin*. 2017;9:537-549.



European  
Reference  
Network

for rare or low prevalence  
complex diseases

Network  
Heart Diseases  
(ERN GUARD-HEART)



EUROPEAN REFERENCE NETWORKS  
FOR RARE, LOW-PREVALENCE AND COMPLEX DISEASES

Share. Care. Cure.



European  
Reference  
Networks



European  
Commission | Health

ERN GUARD-Heart  
Project  
Management  
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